A Phase 1 Study of ECT-001 Expanded Cord Blood and Myeloablative Regimen with Reduced Toxicity in Patients with Severe Sickle Cell Disease.

Grant Award Details

A Phase 1 Study of ECT-001 Expanded Cord Blood and Myeloablative Regimen with Reduced Toxicity in Patients with Severe Sickle Cell Disease.

Grant Type: Cure Sickle Cell Initiative Clinical Trial Stage Projects

Grant Number: CLIN2SCD-11674

Project Objective: The objective is to complete a Phase 1 clinical trial of HSC transplantation of ECT-001-expanded cord blood HSC in patients with severe Sickle Cell Disease.

Investigator:

Name:	Pierre Caudrelier
Institution:	ExCellThera Inc.
Type:	PI

Disease Focus: Blood Disorders, Sickle Cell Disease

Human Stem Cell Use: Adult Stem Cell

Award Value: $2,000,000

Status: Active

Grant Application Details

Application Title: A Phase 1 Study of ECT-001 Expanded Cord Blood and Myeloablative Regimen with Reduced Toxicity in Patients with Severe Sickle Cell Disease.

Public Abstract:

Therapeutic Candidate or Device

ECT-001 graft contains more stem and immune cells than conventional cord blood graft, leading to prompt recovery and better outcomes for patients.

Indication

Severe Sickle Cell Disease

Therapeutic Mechanism

Hematopoietic stem cell transplantation is the only cure for severe sickle cell disease. The ECT-001 expanded cord blood cells will replace the patient's sickling red blood cells with healthy cells.

Unmet Medical Need

African-Americans are the most affected by sickle cell disease. Unfortunately, donor availability for this specific population is very limited for standard bone marrow transplantation. Using cord blood unit, with sufficent cell doses, would eliminate the donor availability problem.

Project Objective

Phase 1 Trial completed

Major Proposed Activities

- Manufacture product to supply the proposed trial
- Assess clinical safety and efficacy

Statement of Benefit to California:

Our innovative technology has the potential to greatly increase the quality of life and prevent early mortality for patients with blood disorders, such as leukemia, myeloma and sickle cell disease. 12 California patients will be the first to benefit of this curative technology, at very low costs. Also, Californian clinical sites involved in the trial will develop the expertise on ECT-001 grafts, and will therefore be the first to benefit from it once ECT-001 is approved.

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